a Wish in Motion

EXTERNALLY-LED PATIENT-FOCUSED DRUG DEVELOPMENT MEETING FOR SCN2A-RELATED DISORDERS

PRESENTED BY THE FAMILIESCN2A® FOUNDATION

WASHINGTON, D.C. ✦ OCT 23 ✦ SCN2A.ORG

ARIA Aligning Research to Impact Autism

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RARE FOUNDATION

Friday, October 23, 2026
9:00 am - 4:00 pm ET (in person)
10:00 am - 3:15 pm ET (virtual)
AGU Conference Center
2000 Florida Ave NW,
Washington, DC, USA

Livestream link:
https://us02web.zoom.us/webinar/register/WN_IOmKesFdQrKMGe3grmYnJQ

Discussion Questions

Although we recognize that most people with SCN2A-related disorders are non-verbal, we encourage any self-advocates to answer for themselves whenever possible. Parents and caregivers - please answer questions from the perspective of the person living with the SCN2A-related disorder. Bereaved families are encouraged to participate and modify the questions to feel more inclusive of their experience. Everyone else is in “listen and learn mode” and should not answer questions.

Topic 1: Health Effects and Daily Impacts of SCN2A

1. Of all the symptoms that you experience because of SCN2A, which 1-3 symptoms have the most significant impact on your life?
2. Are there specific activities that are important to you that you cannot do at all (or as fully as you would like) because of SCN2A?
3. Related to SCN2A, what does a good day look like? What does a bad day look like?
4. How has your condition and its symptoms changed over time? Would you define your condition today as being well managed?
5. What worries you most about SCN2A?

Topic 2: Treating SCN2A

6. What are you currently doing to help treat your condition or its symptoms?
7. How well does your current treatment regimen treat the most significant symptoms of your condition?
8. What are the most significant downsides to your current treatments, and how do they affect your daily life?
9. Short of a complete cure, what specific things would you look for in an ideal treatment for SCN2A? What would you consider to be a meaningful improvement in your condition that a treatment could provide?

Topic 3: Clinical Trials for SCN2A

10. If you considered participating, or have participated, in a clinical trial for SCN2A, please tell us about your experience. Was there anything that made it easier or more difficult to participate?
11. If you were deciding whether to join a clinical trial, how important would the following be to you? 1. The treatment being studied, and 2. the trial logistics
12. What results or improvements are most important for a clinical trial to measure?

Polling

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Join the Live Polling!

Scan the QR code or visit menti.com and enter code 3308 1343. Keep the tab open throughout the meeting to answer new questions.

Polling is for self-advocates and family members only. All others are invited to observe and learn. Your responses will help shape the Voice of the Patient Report, and may also be used as part of the SNORKLE Study (SCN2A Narratives, Outcomes, Research, Knowledge & Lived Experience)

Agenda

10:00 - 10:05 **Welcome**
Leah Schust Myers
Executive Director, FamilieSCN2A Foundation

10:05 - 10:10 **Opening Remarks**
Michelle Campbell, PhD
Associate Director for Stakeholder Engagement and Clinical Outcomes, US Food and Drug Administration (FDA), Center for Drug Evaluation and Research (CDER)

10:10 - 10:20 **Clinical Overview**
Laura McGarry, MD PhD
Attending Neurologist, Division of Neurology, Children's Hospital of Philadelphia

10:20 - 10:30 **Overview of Discussion Format**
Kevin Bender, PhD
Professor of Neurology, University of California San Francisco

Audience Polling
Christina SanInocencio, PhD
Assistant Professor of Communication, Fairfield University
SNORKLE Study Principal Investigator

10:30 - 11:20 **Topic 1: Health Effects and Daily Impacts of Living with SCN2A-Related Disorders**
Featured stories (Mark, Isaiah, Isla, Miles, & Charlotte)
Panel of siblings will provide comments to start the discussion

11:20 - 12:00 **Large-Group Facilitated Discussion on Topic 1**
Self-advocates / families in the audience are invited to add to the dialogue

12:00 - 1:00 **Lunch Break & Group Photo**

1:00 - 1:30 **Topic 2: Current and Past Approaches to Treatment**
Featured stories (Logan, Leo, Sophia, & Emily)
Panel of self-advocates will provide comments to start the discussion

1:30 - 2:00 **Large-Group Facilitated Discussion on Topic 2**
Self-advocates / families in the audience are invited to add to the dialogue

2:00 - 2:10 **Break**

2:10 - 2:35 **Topic 3: Perspectives on Clinical Trials for SCN2A Treatments**
Featured stories (Ethan, Tomas, & Carson)
Panel of parents will provide comments to start the discussion

2:35 - 3:00 **Large-Group Facilitated Discussion on Topic 3**

Patients / caregivers in the audience are invited to add to the dialogue.

3:00 - 3:15 **Summary of What We've Learned**

Christina SanInocencio, PhD

Principal Investigator, SNORKLE Study

Closing Remarks

Melody Kisor, MS, BCPA

Director of Advocacy & Education, FamilieSCN2A Foundation

3:15 - 4:00 **Reception**



A heartfelt thank you to our meeting sponsors, and to the FamilieSCN2A Foundation Board of Trustees, staff, dedicated volunteers, and all the families who have shared their voices to make this meeting so meaningful.

Special thanks to the global network of SCN2A Patient Advocacy Groups providing community, support, and leadership to the international community